
Development of a Gene Therapy for the Treatment of Pitt Hopkins Syndrome (PHS) - Translating from Animal Proof of Concept to Support Pre-IND Meeting

Grant Award Details

Development of a Gene Therapy for the Treatment of Pitt Hopkins Syndrome (PHS) - Translating from Animal Proof of Concept to Support Pre-IND Meeting

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-13997

Investigator:

Name:	Allyson Berent
Institution:	Mahzi Therapeutics
Type:	PI

Award Value: \$4,000,000

Status: Pre-Active

Grant Application Details

Application Title: Development of a Gene Therapy for the Treatment of Pitt Hopkins Syndrome (PHS) - Translating from Animal Proof of Concept to Support Pre-IND Meeting

Public Abstract:**Translational Candidate**

MZ-1866 is a recombinant AAV9 based gene therapy containing the transgene encoding Transcription Factor 4 (TCF4)

Area of Impact

Pitt Hopkins Syndrome is a rare genetic neurological disease which causes profound disability and severe health impact

Mechanism of Action

Patients with Pitt Hopkins Syndrome have heterozygous mutations in the Transcription Factor 4 (TCF4) gene resulting in haploinsufficiency. A single delivery of Mz-1866 into the central nervous system may transduce neurons to replace the deficiency of TCF4 and ultimately improve the clinical phenotype of patients.

Unmet Medical Need

Pitt Hopkins Syndrome (PHS) symptoms include severe intellectual disability, delayed motor development, limited/no speech, constipation, autism-like behaviors, breathing problems and seizures. Mz-1866 has the potential to improve symptoms and would be the first disease-modifying treatment for PHS.

Project Objective

The objective is to hold an FDA pre-IND meeting

Major Proposed Activities

- Develop a manufacturing process for a gene therapy and manufacture enough to complete rodent and non-human primate animal studies
- Conduct a pilot safety, tolerability and bio-distribution study of two dose levels of MZ-1866 in non-human primates
- Conduct interviews with patient caregivers to develop a deeper understanding of the disease experience and develop meaningful clinical endpoints.

Statement of Benefit to California:

It's estimated that 1 in 225,000 children are born with Pitt Hopkins Syndrome, which means there may be close to 200 families in California living with this condition. Mahzi, a California company, will collaborate with partner organizations & vendors in our state, including Pitt Hopkins Research Foundation, UC San Diego, Rady Children's Consortium for newborn screening and Endpoint Outcomes. Our efforts will support identification and inclusion of California families in the pursuit of a therapy.

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